

Fact Sheet

The Rare Diseases Clinical Research Network

A rare disease typically affects a patient population estimated at fewer than 200,000 in the U.S. There are more than 6,000 rare diseases known today and they affect an estimated 25 million persons in the U.S.

Yesterday

- In 1989, the National Commission on Orphan Diseases recommended an increase in funding for Clinical Research Centers and criteria of evaluation that would emphasize rare diseases research.
- In 1999 NIH convened the Special Emphasis Panel of the National Institutes of Health on the Coordination of Rare Diseases Research.
- The Panel recommended that NIH should support the establishment of Specialized Research and Diagnostic Centers of Excellence for Rare Diseases.

Today

- The Rare Diseases Act of 2002 enabled the NIH to establish a network of clinical research centers devoted to research on rare diseases.
- In 2003, the National Institutes of Health created the Rare Diseases Clinical Research Network.
- In 2004, NIH expanded the Network to a total of 10 consortia and a Data and Technology Coordinating Center (DTCC).
- The following is a list of the primary institutions in the consortia and the area of rare disease investigation:

Children's National Medical Center, Washington, DC — **Urea Cycle Disorders Consortium**

Baylor College of Medicine, Houston, TX — **Angelman, Rett, & Prader-Willi Syndromes Consortium**

Boston University School of Medicine, Boston, MA — **Vasculitis Clinical Research Consortium**

Cleveland Clinic Foundation, Cleveland, OH — **Bone Marrow Failure Disease Consortium**

Mount Sinai School of Medicine, New York, NY — **RareGenetic Steroid Disorders Consortium**

Children's Hospital Medical Center, Cincinnati, OH — **Rare Lung Diseases**

University of Rochester, Rochester, NY — **Consortium for Clinical Investigations of Neurological Channelopathies (CINCH)**

University of North Carolina at Chapel Hill, NC — **Genetic Diseases of Mucociliary Clearance Consortium**

Children's Hospital, Denver, CO — **Cholestatic Liver Disease Consortium (CLiC)**

Duke University School of Medicine, Durham, NC — **Rare Thrombotic Diseases**

Pediatrics Epidemiology Center, University of South Florida, Tampa, FL — **The Data and Technology Coordinating Center (DTCC)**

- On May 5, 2006, NIH announced the recruiting of patients for the first group of clinical studies of the Network. More than 20 studies are expected to open in the next few months and up to a total of fifty thereafter.
- The Rare Diseases Clinical Research Network accelerates our entry into the age of molecular medicine.

Tomorrow

“By studying the genetic component of these rare diseases, NIH hopes to be able to better predict the course of illnesses and provide more effective, personalized treatments for those afflicted... Ultimately, this individualized approach, completely different from how we treat patients today, will allow us to prevent or to promptly treat the complications arising from these genetic (or acquired) disorders.” – **NIH Director, Elias A. Zerhouni, MD**